outcomes, and study designs). **RESULTS:** A review of the clinical efficacy of ivacaftor, its comparative clinical efficacy compared with dornase alfa, and a review of the cost-effectiveness of ivacaftor will help to inform decisions about the treatment of patients with CF. Four studies were presented as evidence of the benefit of ivacaftor in CF. Two pivotal trials STRIVE and ENVISION, one open label extension study, PERSIST, for patients in STRIVE and ENVISION and a final study in different patient group, DISCOVER, in patients who are homozygous for the F508del mutation. The percent predicted forced expiratory volume in 1 second (FEV1) was the primary outcome measure for the two phase III clinical trials. The review group noted the absence of long term efficacy data particularly in relation to the benefit of ivacaftor in maintaining percent predicted FEV1 and reducing pulmonary exacerbations and the resultant impact on survival rates. The analysis for this extrapolation is based on a number of prediction models that have been published. The disease progression model predicts that the median survival for a patient treated with ivacaftor will be 29.2 years longer as a consequence of taking the drug. **CONCLUSIONS:** Whilst ivacaftor may represent an innovation for the treatment of patients with cystic fibrosis there are significant uncertainties, including the absence of long term health outcome data.

NEUROLOGICAL DISORDERS – Cost Studies

PND2

ECONOMIC BURDEN OF DRUG USE IN PATIENTS WITH ALZHEIMER'S DISEASE AT PHRAMONGKUTKLAO HOSPITAL AND MEDICAL COLLEGE, THAILAND: A 5-YEAR TREND ANALYSIS

Vichaichanakul K[1], Kanchanaphibool I[2]

[1]*Phramongkutklao Hospital and Medical College, Bangkok, Thailand,* [2]*Silpakorn University, NakornPathom, Thailand*

OBJECTIVES: To determine the trend of drug utilization for Alzheimer's disease during 2009 and 2013. **METHODS:** Prescription data of outpatients with Alzheimer's disease in 2009 – 2013 was extracted from the medical care database of the hospital. Quantities and costs of the prescribed drugs were examined using defined daily dose (DDD) for comparisons. **RESULTS:** Number of patients was rather the same number every year ranging from 1,507 to 1,631 patients. For donepezil and memantine, number of DDD per year was increased every year ranging from 7.3–10.0 % to 1.6-14.7% compared to the previous year, respectively. The increasing trends were not found in prescribing of rivastigmine, except for the dramatically increase (34.0%) in 2013. Galantamine was prescribed less in 2010 and 2013 accounted for -9.2 and -18.2%, respectively. Cost of drug use in Alzheimer's disease was $3,211,269 in 2009 and $3,228,454 in 2013 with an increasingly trend. **CONCLUSIONS:** The overall drug use in Alzheimer's disease seems to be a heavy burden of the hospital every year. The rational drug use should be confirmed to make sure to guarantee appropriate use of drug without overusage.

PND3

BEST PRACTICES AND KEY CHALLENGES IN COST-EFFECTIVENESS MODELLING OF MULTIPLE SCLEROSIS THERAPIES

Smith N, Beckerman R

CBPartners, New York, NY, USA

OBJECTIVES: The purpose of this study was to review cost-effectiveness models in multiple sclerosis (MS) to identify accepted methods, key challenges, and best practices. **METHODS:** We searched MEDLINE, Embase, the Health Economics Evaluation Database (HEED), the Cochrane library, and recent HTA agencies' (NICE and SMC) decisions for studies published prior to March 7, 2014. Following duplicate removal, 100 studies were identified. Studies were excluded if they did not estimate cost-effectiveness in MS, were duplicates, or weren't published in English, resulting in a total of 26 studies sourced. A data extraction form was developed to capture information about model characteristics, patient natural history progression, utility estimates, and the author's comments on the modelling methods. **RESULTS:** All studies were published after 2000, with most focused on first-line USA and EU patient populations. The majority of models utilised a cohort Markov model approach, with health states defined by patient expanded disability status scale (EDSS) scores. Health states included either individual or aggregate EDSS scores (0-9.5) for relapsing-remitting MS (RRMS) and secondary progressive MS (SPMS), as well as a death state. Transition probabilities were sourced from trial data for low score EDSS states, while transitions for higher score EDSS states were sourced from a longitudinal study of Canadian MS patients, due to insufficient clinical trial data for patients with advanced disease. Key challenges identified in recent HTA decisions include modelling EDSS score improvement early in disease natural history, patients' initial distributions across EDSS states, extended benefits of therapy, and patient treatment adherence. **CONCLUSIONS:** Established modelling best practice in MS utilises a cohort Markov model approach with health states simulating patient populations via EDSS scores in RRMS and SPMS. Future studies and HTA submissions should focus on more accurately reflecting patient natural history in the early stages of disease.

PND4

TREATMENT REASONS, RESOURCE USE AND COSTS OF HOSPITALIZATIONS IN PEOPLE WITH PARKINSON'S: RESULTS FROM A LARGE RCT

Xin Y[1], Clarke CE[2], Muzerengi S[3], Rick CE[3], Gray A[4], Gray R[4], Wheatley K[3], Ives N[3], McIntosh E[1]

[1]*University of Glasgow, Glasgow, UK,* [2]*University of Birmingham, Sandwell and West Birmingham Hospitals NHS Trust, Birmingham, UK,* [3]*University of Birmingham, Birmingham, UK,* [4]*University of Oxford, Oxford, UK*

OBJECTIVES: Reasons for hospitalisations in people with Parkinson's Disease (PD) are broad ranging and costly, however detailed analysis of hospitalisations in a large, representative group of PD patients is lacking. This study aimed to explore the reasons for, resource use and associated cost of hospital treatment in participants in the PD MED trial. **METHODS:** We retrospectively reviewed hospitalisation data from 2,074 patients with PD who were recruited into the PD MED trial from Nov 2000 to Dec 2009 and followed up for ten years. PD MED is a large-scale, "real-life" randomised controlled trial comparing the effectiveness and cost-effectiveness of PD medications. Patients' demographic characteristics, disease severity, reasons and duration of hospitalisation were analysed. Reasons for hospitalisations were coded based on the International Classification of Disease (ICD-10). **RESULTS:** Of 2,074 patients, at randomisation, median age was 72 years (IQR 66-77), mean duration with diagnosed PD was 2.8 years (median 2, IQR 1-3) and median Hoehn &Yahr score was 2 (IQR 1.5-2.5). Until Oct 2011, 29% (597/2074) of patients had a total of 941 hospitalisation records. Mean length of stay was 21days (median 9, IQR 3-24). Hospitalisation reasons were classified into 11 categories of PD related conditions and 15 PD unrelated categories. 64.1% of the hospitalisation records can be associated with PD. Main reasons for hospitalisation were: infections including pneumonia and urinary tract infection (18.4%), falls and fractures and other injuries (15.3%), cardiovascular and circulatory disorders (8.7%), central nervous system and disorders of sense organs (8.2%), gastrointestinal disorders (8.0%), and mental health disorders (6.9%). **CONCLUSIONS:** PD related conditions have a significant and broad ranging specialty impact on hospitalisation rates and associated health care costs are substantial. This paper provides economic justification for investing in interventions that manage infection and prevent falls in people with PD.

NEUROLOGICAL DISORDERS – Patient-Reported Outcomes & Patient Preference Studies

PND5

ALZHEIMER'S DISEASE CAREGIVER BURDEN IN JAPAN AND THE 5 E.U

Gupta S[1], Fukuda T[2], Okumura Y[3], Stankus AP[1]

[1]*Kantar Health, Princeton, NJ, USA,* [2]*National Institute of Public Health, Saitama, Japan,* [3]*Institute for Health Economics and Policy, Tokyo, Japan*

OBJECTIVES: Alzheimer's disease (AD) is a chronic and progressive disease that is a significant burden on caregivers. Research indicates AD caregiver burden on health status; but there is limited research on caregiver burden in Japan. The objectives of this study were to examine Japan AD caregiver burden vs. Japan non-caregivers and 5E.U. AD caregivers. **METHODS:** Data were obtained from the 2012 Japan (N=30,000) National Health and Wellness Survey (NHWS) and 2013 5E.U. (UK, Germany, France, Italy, Spain; N=62,000) NHWS, administered online to a representative adult sample (18+ years). Respondents reported on health status (SF-36v2), activity impairment, health care utilization in the past six months and caregiver responsibilities. Multivariable regressions, adjusting for demographics and health history variables to explore differences between Japan AD caregivers (n=714) vs. Japan non-caregivers (n=27,702) and 5E.U. AD caregivers (n=1,239). **RESULTS:** Japan AD caregivers were older and reported more depression symptoms than Japan non-caregivers ($p<0.05$). Japan AD vs. 5E.U. AD caregivers were older and more educated ($p<0.05$). After adjustments, Japan AD caregivers had lower health status ($p<0.001$), higher health care utilization, and greater activity impairment ($p<0.001$) than Japan non-caregivers. Japan AD vs. 5E.U. AD caregivers had better mental (45.7 vs. 43.8, $p<0.001$) and physical (51.0 vs. 50.0, $p=0.021$) health status, marginally less activity impairment (24.5% vs. 27.1%, $p=0.070$), but more health care provider visits (7.6 vs. 5.4, $p<0.001$) and hospitalizations ($p<0.001$). Japan AD caregivers vs. 5E.U. caregivers were less likely to make treatment decisions and manage finances for AD relative ($p<0.001$), were marginally less involved in helping with daily activities (transportation, meals, shopping, $p=0.054$), but no difference was found on bathing/grooming involvement. **CONCLUSIONS:** Japan AD caregivers report more burden including more depression symptoms than Japan non-caregivers. Japan AD caregivers report greater health care utilization than 5E.U. AD caregivers, but report better health status, and less involvement in treatment and finance decisions.

PND6

THE IMPACT OF MULTIPLE SCLEROSIS SEVERITY ON QUALITY OF LIFE, STRESS, DEPRESSION AND SOCIAL SUPPORT NEEDS

Colman S[1], O'Leary BA[2], Palmer AJ[3], Simmons R[4]

[1]*Covance Pty Ltd, North Ryde, Australia,* [2]*Covance Pte Ltd., North Ryde, Australia,* [3]*University of Tasmania, Hobart, Australia,* [4]*Canberra Hospital, Canberra, Australia*

OBJECTIVES: Multiple Sclerosis (MS) is a chronic disease which results in increasing disability over time. The Australian Multiple Sclerosis Longitudinal Study (AMSLS) is an ongoing study that collects information on around 3,100 volunteers with MS from all States and Territories in Australia. The WHO quality of life assessment instrument (WHOQOL-100) was collected as well as the following Patient Reported Outcome (PRO) measures; MS Self-Efficacy Scale, Perceived Stress Scale, Geriatric Depression Scale – short version (GDP-5), Social Support Scale, Therapeutic Self-Care Scale, and the Depression Anxiety and Stress Scale (DASS). In this analysis, we quantified the difference in utility, stress, depression and social support needs between disease severities in subjects with MS. **METHODS:** Data from the WHOQOL-100 were collected in 2008. The utility score was calculated by mapping five questions from the WHOQOL-100 to the EQ-5D descriptive system as described by Al-Ruzzeh et al (2008). The UK TTO value set (utility weights) were applied to each of the levels in each dimension. Disease severity was based on the self reported Disease Steps Scale. **RESULTS:** A total sample of 2139 subjects provided evaluable data; a response rate of approximately 70%. Overall average QOL as measured by the WHOQOL-100 was 13.7 (95%CI: 13.5 to 13.8) out of a maximum value of 20, ranging from 11.8 (95%CI: 11.4 to 12.2) in severe disease to 15.2 (95%CI: 15.0 to 15.4) when mild. The utility score for all people with MS was 0.65 (95%CI: 0.64 to 0.67). The utility decreased with increasing disease severity with values of 0.80 (95%CI: 0.78 to 0.81), 0.60 (95%CI: 0.58 to 0.61) and 0.42 (95%CI: 0.39 to 0.46) for mild, moderate and severe disease, respectively. The other instruments on the whole followed this trend. **CONCLUSIONS:** Higher disease severity in subjects with MS is associated with lower utility and QOL and worse outcomes in general.